Hong Kong Exchanges and Clearing Limited and The Stock Exchange of Hong Kong Limited take no responsibility for the contents of this announcement, make no representation as to its accuracy or completeness and expressly disclaim any liability whatsoever for any loss howsoever arising from or in reliance upon the whole or any part of the contents of this announcement.

CANbridge Pharmaceuticals Inc.
北海康成製藥有限公司
(Incorporated in the Cayman Islands with limited liability)
(Stock Code: 1228)

INSIDE INFORMATION

This announcement is made pursuant to Rule 13.09 of the Rules Governing the Listing of Securities on The Stock Exchange of Hong Kong Limited and Part XIVA of the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong).

CANbridge Pharmaceuticals Inc. (the "**Company**", together with its subsidiaries, the "**Group**") hereby informs the shareholders and potential investors of the Company of the attached press release that the Group has published the initial data the from its gene therapy research agreement with the Horae Gene Therapy Center, at the UMass Chan Medical School, at the ASGCT 25th Annual Meeting.

The Group cannot guarantee that the novel second-generation scAAV9 gene therapy published in the aforesaid press release will ultimately be successfully developed and marketed. Shareholders and potential investors of the Company are advised to exercise caution when dealing in the shares of the Company.

By Order of the Board of Directors
CANbridge Pharmaceuticals Inc.
Dr. James Qun Xue
Chairman

Beijing, 17 May 2022

As at the date of this announcement, the board of directors of the Company comprises Dr. James Qun Xue as executive director; Dr. Kan Chen, Dr. Derek Paul Di Rocco and Mr. Xiao Le as non-executive directors; and Mr. James Arthur Geraghty, Dr. Richard James Gregory, Mr. Peng Kuan Chan and Dr. Lan Hu as independent non-executive directors.

CANbridge-UMass Chan Medical School Gene Therapy Research Presented at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

First Study from CANbridge UMass Collaboration Shows Novel Gene Therapy Holds Promise for Spinal Muscular Atrophy Clinical Applications

BEIJING and CAMBRIDGE, Mass., May 16, 2022 – CANbridge Pharmaceuticals Inc. (HKEX:1228), a leading China-based global biopharmaceutical company committed to the research, development and commercialization of transformative rare disease and rare oncology therapies, announced the presentation of the initial data from its gene therapy research agreement with the Horae Gene Therapy Center, at the UMass Chan Medical School, at the ASGCT 25th Annual Meeting, in Washington DC, today.

In work led by Jun Xie Ph.D., in the lab of Guangping Gao, Ph.D., researchers concluded that a novel second-generation scAAV9 gene therapy, expressing co-hSMN1 from an endogenous hSMN1 promoter, demonstrated superior potency, efficacy and safety in mice with spinal muscular atrophy (SMA), compared to the benchmark vector, scAAV9-CMVen/CB-hSMN1, which is similar to the vector used in the gene therapy approved by the US Food and Drug Administration for the treatment of SMA.

The novel second-generation gene therapy showed superior efficacy to the benchmark vector in SMA mice along several endpoints, including extended lifespan, restored muscle function and better neuromuscular junction innervation, without the liver toxicity shown in the benchmark-treated animals. Specifically, the second-generation gene therapy significantly extended the lifespan of SMA mice in a dose-dependent manner, with all doses showing improved survival, compared to both the benchmark gene therapy vector high dose and to untreated SMA mice. The second-generation gene therapy also restored muscle function in SMA mice significantly better than the benchmark vector. This was observed in both the righting test, in which second-generation gene therapy-treated SMA mice were able to right themselves faster than the benchmark vector-treated mice, and in the grid test, in which they demonstrated better muscle function. In addition, the second-generation vector restored the innervation of the neuromuscular junctions in SMA mice to close to that of wild-type mice, and significantly better than in SMA mice treated with the benchmark vector.

Finally, SMA mice treated with the second-generation gene therapy showed higher SMN1 expression in the central nervous system and lower peripheral tissue than the benchmark vector-treated mice, in a pattern that was similar to that of healthy carrier mice. Furthermore, the benchmark vector produced liver damage in four out of seven SMA mice, eight days post-injection, compared to no liver toxicity in mice treated with the second-generation gene therapy vector, or in healthy carrier mice, suggesting that the second-generation gene therapy has the potential to reduce liver toxicity and overcome current therapeutic limitations.

This is the first data to be presented from the gene therapy research collaboration between CANbridge and the Gao Lab at the Horae Gene Therapy Center.

“What differentiates our novel second-generation gene therapy vector from the benchmark vector is the genetic engineering of a codon-optimized SMN1 transgene under the control of the endogenous SMN1 promoter, which enables highly efficient and regulated gene expression across tissues, with the potential to improve both efficacy and safety, while at a lower dose than is currently used in patients,” said Yunxiang Zhu, Ph.D., Vice President, Head of Global Research, CANbridge Pharmaceuticals, and a study author. “These data encourage us to support the continued development of this second-generation vector as a potential best-in-class gene therapy for SMA.”

"We are seeking to develop a next-generation gene therapy for SMA that leverages the advances in gene therapy that have occurred since the first gene therapy was developed, over a decade ago," said Guangping Gao, Ph.D., Co-Director, Li Weibo Institute for Rare Diseases Research, Director, the Horae Gene Therapy Center and Viral Vector Core, Professor of Microbiology and Physiological Systems and Penelope Booth Rockwell Professor in Biomedical Research at UMass Chan Medical School, and a lead study author. Dr. Gao is also a former ASCGT president.

Presentation Details:

Title: Endogenous Human SMN1 Promoter-driven Gene Replacement Improves the Efficacy and Safety of AAV9-mediated Gene Therapy for Spinal Muscular Atrophy in Mice

Poster #: M-144

Category: Neurologic Diseases: AAV Vectors – Preclinical and Proof-of-Concept Studies

Category: Neurologic Diseases I

Session Date and Time: Monday, May 16, 5:30-6:30 PM EDT

Authors: Qing Xie, Hong Ma, Xiupeng Chen, Yunxiang Zhu, Yijie Ma, Leila Jalinous, Qin Su, Phillip Tai, Guangping Gao, Jun Xie

Abstracts are available on the ASGCT website: https://annualmeeting.asgct.org/abstracts

About the Horae Gene Therapy Center at UMass Chan Medical School

The faculty of the Horae Gene Therapy Center is dedicated to developing therapeutic approaches for rare inherited disease for which there is no cure. We utilize state of the art technologies to either genetically modulate mutated genes that produce disease-causing proteins or introduce a healthy copy of a gene if the mutation results in a non-functional protein.

The Horae Gene Therapy Center faculty is interdisciplinary, including members from the departments of Pediatrics, Microbiology & Physiological Systems, Biochemistry & Molecular Pharmacology, Neurology, Medicine and Ophthalmology. Physicians and PhDs work together to address the medical needs of rare diseases, such as Alpha 1-Antitrypsin Deficiency, Canavan Disease, Tay-Sachs and Sandhoff diseases, Retinitis Pigmentosa, Cystic fibrosis, Lou Gehrig's disease, TNNT1 nemaline myopathy, Rett syndrome, N-Gly 1 deficiency, Pitt-Hopkins syndrome, Marple Syrup Urine Disease, Sialidosis, GM3 synthase deficiency, Huntington's disease, ALS and others. More common diseases such as cardiac arrhythmia and hypercholesterolemia are also investigated. The hope is to treat a wide spectrum of diseases by various gene therapeutic approaches. Additionally, the University of Massachusetts Chan Medical School conducts clinical trials on site and some of these trials are conducted by the investigators at the Gene Therapy center.

About CANbridge Pharmaceuticals Inc.

CANbridge Pharmaceuticals Inc. (HKEX:1228) is a China-based global biopharmaceutical company committed to the research, development and commercialization of transformative therapies for rare disease and rare oncology. CANbridge has a differentiated drug portfolio, with three approved drugs and a pipeline of 11 assets, targeting prevalent rare disease and rare oncology indications that have unmet needs and significant market potential. These include Hunter syndrome and other lysosomal storage disorders, complement-mediated disorders, hemophilia A, metabolic disorders, rare cholestatic liver diseases and neuromuscular diseases, as well as glioblastoma multiforme. CANbridge is also building next-generation gene therapy development capability through a combination of collaboration with world-leading researchers and biotech companies and internal capacity. CANbridge global partners include: Apogenix, GC Pharma, Mirum, Wuxi Biologics, Privus, the UMass Chan Medical School and LogicBio.

For more on CANbridge Pharmaceuticals Inc., please go to: www.canbridgepharma.com.

Forward-Looking Statements

The forward-looking statements made in this article relate only to the events or information as of the date on which the statements are made in this article. Except as required by law, we undertake no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise, after the data on which the statements are made or to reflect the occurrence of unanticipated events. You should read this article completely and with the understanding that our actual future results or performance may be materially different from what we expect. In this article, statements of, or references to, our intentions or those of any of our Directors or our Company are made as of the date of this article. Any of these intentions may alter in light of future development.

Contact:

U.S. Investor Relations:
Chris Brinzey
ICR Westwicke
Chris.brinzey@westwicke.com

China Investor Relations
CANbridge Pharmaceuticals Inc.
ir@canbridgepharma.com

Media:
Deanne Eagle
Planet Communications
deanne@planetcommunications.nyc
917.837.5866